

## CIRM Funded Clinical Trials

### A Phase I, Open-Label Study To Assess The Safety, Feasibility and Engraftment of Zinc Finger Nucleases (ZFN) CCR5 Modified Autologous CD34+ Hematopoietic Stem/Progenitor Cells (SB-728MR-HSPC) with Escalating Doses of Busulfan In HIV-1 (R5) Infected Subjects with Suboptimal CD4 Levels on cART

<b>Disease Area:</b>	HIV/AIDS
<b>Investigator:</b>	John Zaia
<b>Institution:</b>	City of Hope, Beckman Research Institute
<b>CIRM Grant:</b>	SP3A-07536
<b>Award Value:</b>	\$5,583,438
<b>Trial Sponsor:</b>	Beckman Research Institute of City of Hope
<b>Trial Stage:</b>	Phase 1
<b>Trial Status:</b>	Recruiting
<b>Targeted Enrollment:</b>	12
<b>ClinicalTrials.gov ID:</b>	NCT02500849



John Zaia

#### Details:

A team at City of Hope and Sangamo Therapeutics is genetically modifying patients' blood forming stem cells to functionally cure people with HIV. The team is using a technology called zinc finger nucleases – a kind of molecular scissors – to snip out the target gene that codes for the CCR5 receptor. This receptor is the gateway for the HIV virus into immune cells. The hope is that this treatment will provide a renewable, long-lasting source of HIV resistant immune cells in patients.

#### Design:

Single arm, non-randomised study.

#### Goal:

Safety. Efficacy - engraftment.

#### Updates:

Enrolling.

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